Rare Disease Legislative Advocates
May Legislative Meeting

May 17, 2016
1. **Medicines in Development Report on Rare Diseases**: Holly Campbell, Senior Director of Communications, PhRMA

2. **AB 2752 (California) Continuity of Care**: Jerry Jeffe, Public Policy Director, California Chronic Care Coalition

3. **Lymphedema Treatment Act**: Patricia Egan, Interim Executive Director & CEO, National Lymphedema Network

4. **SB 1095 (California) Newborn Screening**: Vignesh Ganapathy, Manager of Advocacy and Government Relations, EveryLife Foundation for Rare Diseases

5. **Capitol Hill Update**: Max Bronstein, Senior Director of Advocacy and Science Policy, EveryLife Foundation for Rare Diseases
Medicines in Development Report on Rare Diseases

Holly Campbell
Senior Director of Communications
PhRMA

May 17th, 2016
AB 2752 (California)
Continuity of Care

Jerry Jeffe
Public Policy Director
California Chronic Care Coalition

May 17th, 2016
California Assembly Bill 2752 (Nazarian) for Health Care Coverage: Continuity of Care

Bill Sponsored by the California Chronic Care Coalition (www.chroniccareca.org)

Assembly Bill 2752 does two things:
(1) Beginning January 1, 2017, health plans will have to notify enrollees of prescription drug formulary changes in annual renewal materials, and
(2) Beginning January 1, 2017 health plans will have to provide information regarding their provider directories in annual renewal materials.
Reason for AB 2752:
Consumers are often unaware of formulary changes in their health plans. This will require health plans to notify consumers before they renew their plans for another year if their prescription drugs are dropped from the plan. This is especially important for people who live with chronic disease.

Status:
Bill passed Assembly Health Committee, 14-3, with members of both parties voting yes. Bill is now on the Suspense File in Assembly Appropriations Committee. The committee will most likely announce which bills go off Suspense and to the Assembly Floor on May 27.

Action Request:
Send your name, email address, and organization, if any, to info@chroniccareca.org. You will be sent information on the best ways to contact the committee and urging its members to ask that AB 2752 be sent to the Assembly Floor.

Information:
Copy of bill and committee analyses can be downloaded at www.assembly.ca.gov. Questions: Contact Jerry Jeffe, California Chronic Care Coalition, 916-502-7734 (Cell) or jerry.jeffe@gmail.com.
Lymphedema Treatment Act

HR 1608/S 2373
The Lymphedema Treatment Act provides a Medicare benefit to cover essential compression materials (garments, bandages, etc.) needed for treatment and daily care management of lymphedema.

Lymphedema refers to several rare diseases. See NORD http://rarediseases.org/rare-diseases/hereditary-lymphedema/
Bipartisan and bicameral, the Lymphedema Treatment Act was introduced in the 114th Congress as:

- HR 1608 by Rep David Reichert (R-WA-8) March 2015
- S 2373 by Senator Maria Cantwell (D-WA) December 2015
LYMPHEDEMA TREATMENT ACT STATUS

• 225 cosponsors in the House
• 16 cosponsors in the Senate

Rep. David Reichert (R-WA-8) sent press release
50+% of House now cosponsors this bill.
THANK YOU, RDLA, for supporting the Lymphedema Treatment Act!

www.LymphedemaTreatmentAct.org

Patricia Egan, MS, MBA
Interim Executive Director & CEO
National Lymphedema Network
California State Team Leader
Lymphedema Advocacy Group
Patricia.Egan@lymphnet.org
California SB 1095: Newborn Screening

Mike Carpenter
EveryLife Foundation for Rare Diseases
• A patchwork of screening and state’s requiring legislation to add new screening tests
• Babies are falling through the gaps and major delay between development of therapy and implementation of screening
• NBS is vital to ensure early diagnosis and early treatment -> optimal health outcomes
• Launched legislation in California to automatically require the state to screen for all diseases on the RUSP
Newborn Screening Saves Lives & Money

- Early detection and treatment can help avert costly medical procedures (e.g. hospitalizations and surgeries)
- E.g. 1 spinal fusion surgery can cost around $100K
- In the U.S. states spend millions to provide mental health services to children
- In California, every $1 spent on screening = savings of $9.32*

The Advisory Committee on Heritable Disorders

Advisory Committee on Heritable Disorders in Newborns and Children

Advisory Committee on Heritable Disorders in Newborns and Children

Secretarial Responses Regarding the Committee's Recommendations to add Mucopolysaccharidosis Type I (PDF - 159 KB) and Adrenoleukodystrophy (X-ALD) (PDF - 137 KB).

2016 Meetings
- May 9-10, 2016 In-person (Location: Rockville, MD) and Via Webcast
- August 25 and 26, 2016 (Location: Rockville, MD) and Via Webcast
- November 3 and 4, 2016 (webinar)

The Advisory Committee on Heritable Disorders in Newborns and Children (Committee) was established under the Public Health Service Act, Title XI, § 1109 (42 U.S.C. 300b-10), as amended by the Newborn Screening Saves Lives Reauthorization Act of 2014 (P.L. 113-240).
The Process in a Nutshell

- Nominate a disease -> Considered by Committee -> Committee Recommends Addition to Secretary of HHS -> If Accepted, Disease is Added to the RUSP
- Timing: On average, it takes almost 4 years to add a disease to the RUSP
- States take up to 8 years to actually begin screening
### New Additions:
- MPS 1
- X-ALD

### Potential Diseases to be added in the future:
- Duchenne Muscular Dystrophy
- MLD
- Additional MPS diseases
- Batten’s Disease

You can find the full list at [http://www.hrsa.gov/advisorycommittees/mchbadvisory/heritabledisorders/recommendedpanel/](http://www.hrsa.gov/advisorycommittees/mchbadvisory/heritabledisorders/recommendedpanel/)
California

- Screens 540,000 newborns each year for all but TWO of the RUSP conditions:
  - Pompe
  - MPS I
- Both have had FDA-approved therapies for over a decade
- Has 5 public health laboratories currently using MS/MS of dried blood spots
- In 2014, passed bill screening for Adrenoleukodystrophy (ALD) when added to the RUSP
• Introduced by State Senator Richard Pan
  – Pediatrician who authored lifesaving ALD and SCID newborn screening legislation
SB 1095

• Would allow state to screen for a disease as soon as it is added to the RUSP

• Would eliminate legislative delay in screening
  – Can take up to eight years before a state begins screening for a disease after its added to the RUSP
  – No need to pass new legislation for every disease

• Takes scientific approach instead of political

• Would allow for earliest treatment possible, saving many patients lives
SB 1095

- Must pass two committees in each house:
  - Health
  - APPROPRIATIONS
- Need economic data to show that screening for MPS I and Pompe will save the state money
  - Search for diagnosis
  - Surgeries & hospital stays
  - Mental health & developmental care
SB 1095

- SB 1095 passed the Senate Health Committee unanimously!
- Advocates from across the country came and testified
Important Upcoming Dates

• May 27th: Last day for SB 1095 to be heard in Senate Appropriations Committee
• June 3rd: Last day for Senate Floor vote
• July 1st: Last day for SB 1095 to be heard in Assembly Health Committee
• July 1st-August 1st: Summer Recess
• August 12th: Last day for SB 1095 to be heard in Assembly Appropriations Committee
• August 31st: Last day for Assembly Floor vote
• September 30th: Last day for governor to sign or veto
How YOU can help

Sign-On to Support Newborn Screening

The EveryLife Foundation for Rare Diseases is proud to sponsor and support SB 1095 (Pan), which would improve the health of babies born in California by expanding newborn screening to include diseases on the federal Recommended Uniform Screening Panel (RUSP). The Secretary of the United States Department of Health and Human Services, convenes a committee of newborn screening experts to develop the RUSP. The federal process is rigorous, evidence-based, and science-driven, but ultimately states are not required to follow its recommendations. This results in babies in some states receiving timely diagnosis and treatment, while babies born in other states do not, with potentially life-threatening consequences.

• Help provide case studies of the benefits of late vs. early treatment

• Join the sign-on letter, currently over 80 endorsing national patient organizations including March of Dimes, NORD, Muscular Dystrophy Association & Global Genes!

• Share this link with your networks

www.EveryLifeFoundation.org
OR
bit.ly/1RK4HFR
How YOU can help

• If you or your organization are based in California:
  – Write letters to your state senators and assembly members
  – Come to Sacramento and support SB 1095 in person!
Capitol Hill Update

Max Bronstein, Senior Director, Advocacy & Science Policy
EveryLife Foundation for Rare Diseases
Push for 21st Century Cures

- The 21st Century Cures Act (HR 6) includes a variety of provisions of critical importance to the rare disease community
- Passed by the House in July 2015 – 344-77
Chairman Alexander Announces Committee Schedule for “Step by Step” Consideration of Biomedical Innovation Bills

Feb. 9 meeting will be 1st of 3 to produce companion legislation to 21st Century Cures Act already passed by House

“The House has completed its work on the 21st Century Cures Act. The president has announced his support for a precision medicine initiative and a cancer ‘moonshot.’ It is urgent that the Senate finish its work and turn into law these ideas that will help virtually every American.”

WASHINGTON, D.C., Jan. 19 – Chairman Lamar Alexander (R-Tenn.) today announced the Senate health committee will hold its first executive session considering bills on biomedical innovation on Tuesday, Feb. 9. At that committee
Rare Disease Provisions:

February 9th:
- Advancing Targeted Therapies for Rare Diseases Act of 2015 (Sens. Bennet, Burr, Warren, and Hatch)
- Advancing Neurological Diseases Act of 2015 (Sens. Isakson & Murphy) – provision added that would limit surveillance to the five most prevalent diseases
- All bills passed by unanimously by voice vote

March 9th:
- Advancing Hope Act of 2015 (Sens. Casey, Isakson, Brown & Kirk)
- Legislation Supporting Precision Medicine Initiative
- Patient Focused Impact Assessment (PFIA) Act (Sens. Wicker, Klobuchar, Collins, Franken, Isakson, Bennet, Donnelly)

April 6th:
- Workforce Enhancement at NIH & FDA
Missing Rare Disease Provisions

- No Agreement (yet) on NIH/FDA Funding
- No language on Compassionate Use
- Orphan Product Extensions Now, Accelerating Cures & Treatments Act (OPEN ACT) – Repurposing Incentive (Sens. Hatch & Klobuchar)
NIH & FDA Funding: Deal or No Deal?

Passed the House = $10B in spending
- NIH: $1.8B/year 5 years, mandatory funding
- FDA: $110M/year 5 years, mandatory

Proposed in the Senate = R’s – $8.8B, D’s – $50B in spending
- NIH & FDA: $5B/year for 10 years, mandatory

- Dems have stated that they will not support legislation unless an agreement can be reached on NIH/FDA funding
- Some Republicans are concerned about using mandatory funding
- Leader McConnell has stated, if a deal is made, bill will get floor time
- Some in Senate have hinted that legislation will move in June, others have hinted at a longer timeline through 2017
Assuming a deal is made....
Take ACTION Today

➤ TODAY: E-Action Alert

➤ STAY TUNED: Phone Action Alerts for Floor Vote

➤ MAXIMUM IMPACT: Meet with your representatives in Congress – Especially: Washington State & Tennessee

www.EveryLifeFoundation.org
Wednesday on Capitol Hill

RARE DISEASE CONGRESSIONAL CAUCUS

BRIEFING:

THE NIH AND FDA:
VITAL AGENCIES IN THE FIGHT AGAINST RARE DISEASES

WEDNESDAY, MAY 18TH, 2016
2:00 - 3:00 PM
SENATE CAPITOL VISITOR CENTER
ROOM 201
Moderator: Ellie Dehoney, Vice President of Policy and Advocacy, Research!America

- The Undiagnosed Disease Program at the NIH
  - William Gahl, M.D., Ph.D, Clinical Director, National Human Genome Research Institute
- Precision Medicine – The White House & the FDA
  - Matthew Might, Strategist, Executive Office of the President, The White House, Associate Professor, University of Utah, Associate Professor, Visiting, Harvard Medical School, Founder, NGLY1.org
- The Importance of the FDA Orphan Products Grant Program
  - Marina Cuchel, M.D., Ph.D, Research Associate Professor, Perelman School of Medicine at the University of Pennsylvania
- The Value of Patients to Clinical Innovation at the NIH
  - Kayla Martinez & Dorelia Rivera, NOMID Patient
- The Role of NIH Funding in Kickstarting Biomedical Innovation
  - Christopher C. Gibson, Ph.D, Co-Founder & CEO, Recursion Pharmaceuticals
Thanks for listening

- Questions?
- mbronstein@everylifefoundation.org
Questions?

For all the information listed on this presentation go RareAdvocates.org

Contact Information:

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www.EveryLifeFoundation.org www.RareAdvocates.org
Next Meeting:

Webinar and Conference Call

**June 22nd, 1:00 PM EDT**

If you would like to add an item to the agenda for any future RDLA meetings please send requests to:

vganapathy@everylifefoundation.org